EMA/706927/2022

European Medicines Agency decision

P/0375/2022

of 9 September 2022

on the agreement of a paediatric investigation plan for ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5), (EMEA-003137-PIP01-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Holostem Terapie Avanzate s.r.l. on 18 October 2021 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 22 July 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5), living tissue equivalent, implant use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

This decision is addressed to Holostem Terapie Avanzate s.r.l., Via Glauco Gottardi, 100, 41125 – Modena, Italy.

EMA/461778/2008
Amsterdam, 22 July 2022

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-003137-PIP01-21

Scope of the application

Active substance(s):

Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5)

Condition(s):

Treatment of epidermolysis bullosa

Pharmaceutical form(s):

Living tissue equivalent

Route(s) of administration:

Implant use

Name/corporate name of the PIP applicant:

Holostem Terapie Avanzate s.r.l.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Holostem Terapie Avanzate s.r.l. submitted for agreement to the European Medicines Agency on 18 October 2021 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 22 November 2021.

Supplementary information was provided by the applicant on 25 April 2022. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of epidermolysis bullosa

2.1.1. Indication(s) targeted by the PIP

Treatment of intermediate LAMB3-dependent junctional epidermolysis bullosa

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Living tissue equivalent

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 - HTA-HG5-02** Open-label, single-arm, intra-patient controlled trial to evaluate efficacy and safety of Hologene 5 in children from birth to less than 18 years of age (and adults) with junctional epidermolysis bullosa
Extrapolation, modelling and simulation studies	Not applicable
Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By October 2024
Deferral for one or more measures contained in the paediatric investigation plan:	No